

Proposed Decision Memo for Cochlear Implantation (CAG-00107N)

Decision Summary

The Centers for Medicare and Medicaid Services (CMS) is seeking public comment on the following proposed conclusions:

The evidence is adequate to conclude that cochlear implantation is reasonable and necessary for treatment of bilateral pre-or-postlinguistic, sensorineural, moderate-to-profound hearing loss in individuals who demonstrate limited benefit from amplification. Limited benefit from amplification is defined by test scores of $\leq 40\%$ correct in the best-aided listening condition on tape recorded tests of open-set sentence cognition.

The evidence is sufficient to conclude that a cochlear implant is reasonable and necessary for individuals with hearing test scores of $> 40\%$ and $\leq 60\%$ only when the provider is participating in and patients are enrolled in either an FDA-approved category B IDE clinical trial, a trial under the CMS Clinical Trial Policy, or a prospective, controlled comparative trial approved by CMS as consistent with the evidentiary requirements for National Coverage Analyses (See Appendix B) and meeting specific quality standards (See Appendix C).

CMS is requesting public comments on this proposed decision memorandum pursuant to Section 731 of the Medicare Modernization Act. After considering the public comments and any additional evidence, we will make a final determination and issue a final coverage decision memorandum.

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Proposed Decision Memo

To: Administrative File: CAG 00107N Cochlear Implantation

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Subject: Proposed Decision: Cochlear Implantation

Date: January 7, 2005

I. Proposed Decision

The Centers for Medicare and Medicaid Services (CMS) is seeking public comment on the following proposed conclusions:

The evidence is adequate to conclude that cochlear implantation is reasonable and necessary for treatment of bilateral pre-or-postlinguistic, sensorineural, moderate-to-profound hearing loss in individuals who demonstrate limited benefit from amplification. Limited benefit from amplification is defined by test scores of $\leq 40\%$ correct in the best-aided listening condition on tape recorded tests of open-set sentence cognition.

The evidence is sufficient to conclude that a cochlear implant is reasonable and necessary for individuals with hearing test scores of $> 40\%$ and $\leq 60\%$ only when the provider is participating in and patients are enrolled in either an FDA-approved category B IDE clinical trial, a trial under the CMS Clinical Trial Policy, or a prospective, controlled comparative trial approved by CMS as consistent with the evidentiary requirements for National Coverage Analyses (See Appendix B) and meeting specific quality standards (See Appendix C).

CMS is requesting public comments on this proposed decision memorandum pursuant to Section 731 of the Medicare Modernization Act. After considering the public comments and any additional evidence, we will make a final determination and issue a final coverage decision memorandum.

II. Background

It is estimated that more than 25 million Americans have hearing loss, including one out of four people older than 65.¹ Hearing loss primarily affecting the external and middle ear is referred to as conductive hearing loss. The type of loss which may be helped by a cochlear implant is known as sensorineural hearing loss or nerve deafness, which results when delicate portions of the inner ear known as hair cells have been damaged and fail to perform their normal function of converting sound waves into electrical current that stimulates the auditory nerve to transmit impulses to the brain, where they are recognized as sound. A cochlear implant, which is an electronic device surgically placed under the skin, bypasses the hair cells and directly transmits sounds through multiple electrodes, which stimulate the auditory nerve. According to the National Institute on Deafness and Other Communication Disorders, since these devices were first approved in 1985, approximately 14,000 individuals in the United States have received them, including about 7000 children.² The implant has 4 basic components: a microphone worn externally behind the ear, which picks up sounds; an external speech processor, which converts sounds to electrical signals; a transmitter and receiver/stimulator which forward the signals; and implanted electrodes, which stimulate the fibers of the auditory nerve.

Patients return to the implanting center after 4 to 5 weeks of post surgery healing to have their speech processor programmed. The patient's age, cognitive skills, and length of deafness are among the factors considered during device programming, which entails selection and fitting of the processing strategy that will be used to translate acoustic stimuli into the electric impulses that will stimulate the auditory nerve. The number of visits needed to accomplish optimum device performance will be influenced by such patient factors as age, previous auditory experience and ability to participate actively in the task. Long-term audiologic follow-up is also necessary as responses to nerve stimulation may change over time.

There are three manufacturers of FDA approved cochlear implants and each approved device has specific labeled indications for its use. The choice of a device for a specific patient is a matter to be decided between an informed patient and the surgeon (otologist/otolaryngologist) and audiologist based on the patient's particular needs and expected outcomes.

III. History of Medicare Coverage

Benefit Category Determination

Medicare is a defined benefit program. An item or service must fall within one or more benefit categories, and not otherwise be excluded by statute from coverage §1812 (scope of Part A); §1832(scope of Part B); §1861(s)(Definition of medical and other services). Cochlear implants are covered as prosthetic devices under §1861(s)(8).

Medicare currently has an NCD for cochlear implants in section 50.3 of the Medicare National Coverage Determinations Manual.

Cochlear implants were first covered for adult Medicare beneficiaries in October 1986, supported by the Office of Health Technology Assessment's "Public Health Service Assessment of Cochlear Implant Devices for the Profoundly Hearing Impaired", dated June 30, 1986. This document concluded that: "Cochlear implantation is considered a safe and efficacious therapy for adult patients with postlingual, profound, bilateral, sensorineural deafness who are stimuable and who lack the unaided residual auditory ability to detect sound." Medicare's initial policy decision provided coverage of cochlear implants for patients at least 18 years of age, whose hearing impairment met those audiologic criteria. Additional requirements included that patients were cognitively able to use auditory clues, were willing to undergo an extended program of rehabilitation, were free from middle ear infection, had an accessible cochlear lumen structurally suited to implantation and were free of lesions in the auditory nerve and acoustic areas of the central nervous system. Medicare coverage was expanded to include children in 1992.

In the period since the original Medicare coverage decision, devices have been improved and there have been gradual changes in the degree of hearing loss for which the Food and Drug Administration (FDA) has approved use of cochlear implants. The National Institutes of Health (NIH) issued a Consensus Statement in 1995 which included updated audiologic criteria for implantation in adults: "Indications in favor of an implant are a severe-to-profound sensorineural hearing loss bilaterally and open-set sentence recognition scores less than or equal to 30 percent under best aided conditions."³ Medicare coverage was revised in 1998 to include the NIH recommended audiologic criteria.

We have had informal contacts with a manufacturer of cochlear implants over the past two years to discuss possible additional changes to coverage to reflect revisions to the audiologic criteria included in the approval of newer devices.

IV. Timeline of Recent Activities

CMS received a request from the Cochlear Americas (formerly Cochlear Corporation) to change Medicare coverage requirements to match FDA labeled indications for approval of implantation of a cochlear device.

July 8, 2004 CMS accepted Cochlear Americas' request for a national coverage determination.

September 9, 2004 Comments from the initial 30-day comment period were posted to the coverage website.

V. FDA Status

One device, the Nucleus 24 Contour, manufactured by the Cochlear Corporation (now called Cochlear America), was approved by the FDA, on November 1, 2000, for patients with test scores of $\leq 50\%$ correct in the ear to be implanted ($\leq 60\%$ in the best aided listening condition). This device contained modifications to the Nucleus 24 device which had been approved on June 25, 1998 for patients with test scores of $\leq 40\%$ in the best aided listening condition. That information as well as the FDA indications for other approved devices may be found on the FDA website at www.fda.gov.

Two other manufacturers of cochlear implants are approved by the FDA for somewhat different audiologic criteria:

The MED-EL COMBI 40+ and PULSARci¹⁰⁰ Systems are approved for “adults eighteen (18) years of age or older who have bilateral, sensorineural hearing impairment and obtain limited benefit from appropriately fitted binaural hearing aids. These individuals typically demonstrate bilateral severe to profound sensorineural hearing loss determined by a pure tone average of 70 dB or greater at 500 Hz, 1000 Hz and 2000 Hz. Limited benefit from amplification is defined by test scores of 40% correct or less in best-aided listening condition on CD recorded tests of open-set sentence recognition (Hearing in Noise Test [HINT] sentences).”

Advanced Bionics’ CLARION Bionic Ear and Hi Resolution Bionic Ear Systems are approved for patients:

- “18 years of age or older
- Severe-to-profound, bilateral sensorineural hearing loss (≥ 70 dB HL)
- Postlingual onset of severe or profound hearing loss
- Limited benefit from appropriately fitted hearing aids, defined as scoring 50% or less on a test of open-set sentence recognition (HINT Sentences)”.

CMS assesses relevant health outcomes, above and beyond the safety and effectiveness regulatory mandate of the FDA. Although a device must receive FDA approval or clearance for at least one indication to be eligible for Medicare coverage, except for a category B device under an investigational device exemption (IDE) clinical trial (60 FR 48417, September 19, 1995), FDA approval/clearance alone does not entitle that device to coverage. The device must fall under a Medicare benefit category and be determined to be reasonable and necessary for the diagnosis or treatment of an illness or injury or to improve the functioning of a malformed body member to be covered by CMS. CMS has the authority to conduct a separate assessment of a device’s appropriateness for Medicare coverage, including whether it is reasonable and necessary specifically for its intended use for Medicare beneficiaries (see e.g., 60 FR 48417, 48420, September 19, 1995). Under a premarket approval application (PMA) review, the FDA determines whether or not there is reasonable assurance of safety and effectiveness for the device’s intended use that is stated in its proposed labeling. Medicare NCDs consider the medical benefit and clinical utility of an item or service in determining whether the item or service is considered reasonable and necessary under the Medicare program. CMS determines whether or not the intervention improves net health outcomes in the Medicare population at least as well as established treatments. Thus, FDA PMA approval by itself is not sufficient for making a determination concerning Medicare coverage.

As we similarly stated in 66 FR 58788, 58797 (November 23, 2001) with regard to FDA 510(k) clearance, "[t]he criteria the FDA uses in making determinations related to substantial equivalency under section 510(k) of the Food, Drug, and Cosmetic Act is significantly different from the scientific evidence we consider in making "reasonable and necessary" determinations under Medicare. FDA does not necessarily require clinical data or outcomes studies in making a determination of substantial equivalency for the purpose of device approval under section 510(k) of the Food, Drug, and Cosmetic Act. Medicare NCDs consider medical benefit and clinical utility of an item or service in determining whether the item or service is considered reasonable and necessary under the Medicare program. Thus, a substantial equivalency approval under section 510(k) of FDA is not sufficient for making determination concerning Medicare coverage."

VI. General Methodological Principles of Study Design

When making national coverage determinations, CMS evaluates relevant clinical evidence to determine whether or not the evidence is of sufficient quality to support a finding that an item or service is reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member (§1862(a)(1)(A) of the Social Security Act.) The critical appraisal of the evidence enables us to determine to what degree we are confident that: 1) the specific assessment questions can be answered conclusively; and 2) the intervention will improve net health outcomes for patients. The general methodological principles of study design utilized in our review of the evidence are in Appendix B.

VII. Evidence

We are providing a summary of the evidence we have considered to date. We will consider additional evidence submitted through the public comment period.

Introduction

When making national coverage determinations, CMS evaluates relevant clinical research studies to determine whether or not the evidence is of sufficient strength to support a finding that an item or service is reasonable and necessary. Methodologists have developed criteria to determine weaknesses and strengths of clinical research. In general, some of the methodological attributes of individual studies associated with stronger evidence include:

- Use of randomization (allocation of patients to either intervention or control group) in order to minimize bias.

- Use of contemporaneous control groups (rather than historical controls) in order to ensure comparability between the intervention and control groups.
- Prospective (rather than retrospective) studies to ensure a more thorough and systematic assessment of factors related to outcomes.
- Large enough sample sizes in studies to 1) make chance an unlikely explanation for what was found; and 2) demonstrate both statistically as well as clinically significant outcomes that can be extrapolated to the Medicare population.
- Masking (blinding) to ensure patients and investigators do not know to which group patients were assigned (intervention or control). This is important especially in subjective outcomes, such as pain or quality of life, where enthusiasm about the intervention and other psychological factors may lead to an improved perceived outcome by either the patient or assessor.
- The measures of clinical effectiveness are appropriate for the condition and include final outcomes such as reduced mortality or improved quality of life. In studies relying on intermediate (surrogate) outcomes, a strong and consistent association between the surrogate outcome and the final outcome has been demonstrated.

Consistent findings across studies of net health outcomes associated with a therapeutic intervention as well as the magnitude of its risks and benefits are also key consideration in the coverage determination process.

Discussion of evidence reviewed

1. Assessment questions

The development of an assessment in support of Medicare coverage decisions is based on the same general question for almost all requests, i.e., “Is the evidence sufficient to conclude that the application of the technology under study will improve net health outcomes for Medicare patients?” The formulation of specific questions for the assessment recognizes that the effect of an intervention can depend substantially on how it is delivered, to whom it is applied, the alternatives with which it is being compared, and the delivery setting. In the case of cochlear implantation in the Medicare population the question is:

- Does cochlear implantation improve health outcomes for those beneficiaries who have bilateral pre-or-postlinguistic sensorineural moderate-to-profound hearing loss demonstrated by hearing test results that are less severe than the current Medicare coverage requirement of an open-set test score $\leq 30\%$ correct in the best-aided listening condition on tape recorded tests of open-set sentence cognition?

In general, the outcome of interest is the percentage correct in a test of open-set sentence discrimination. The amount of improvement in the sentence recognition that is considered significant varied. The FDA used an improvement of 20 percentage points as significant, while the requestor suggested that a 15 percentage point improvement was standard in the industry.

2. External systematic reviews/technology assessments

The American Speech-Language-Hearing Association 2004 “Technical Report: Cochlear Implants” provided an up-to-date review of both the technology and issues to be considered in cochlear implantation. It did not contain specific audiologic criteria for implantation. The Technical Report reviews the similarities and differences between the cochlear implants currently having FDA approval. It makes the point that while device selection may vary depending on the particular center where surgery is to be performed and the preferences of both the surgeon and the patient, outcomes are similar for any of the approved devices.

3. Internal technology assessment

Clinical Considerations

Patients with profound sensorineural hearing loss can benefit from cochlear implantation. Initial development of these devices focused on children and younger adults. More recently, older adults have been successfully implanted with these devices. However, there are some key concerns with the use of these devices in the elderly separately from the younger population. These include the appropriate level of hearing loss prior to implantation, the ability of the elderly to acquire the necessary skills to successfully use the devices, and the differences in results in those that lose their hearing gradually vs in a more rapid fashion.

CMS currently covers these devices when the speech recognition scores are 30% or less. The current request is to expand this coverage from 30% to the FDA labeled indications which vary from 40% to 50% speech recognition depending on the particular device. If the evidence is supportive, a lower level of hearing loss as a criterion for implantation would allow a larger group of the Medicare population to potentially benefit from this technology.

Evidence Review

With its request for revision of Medicare coverage requirements for cochlear implants, Cochlear Americas submitted an original document entitled: "Cochlear Implants in the Elderly: A Review of the Predictive Indicators, Outcomes, and Cost-Effectiveness", which also included an extensive bibliography. We gave particular consideration to the several recent documents that specifically dealt with implantation in older patients. All of these studies reported positive results for older patients receiving cochlear implants. We also specifically looked for information that supported the FDA labeling changes from 30% to 40% or 50% in speech recognition. The data is summarized in Table 1.

We also conducted several literature searches, most recently an Ovid MEDLINE search on December 17, 2004, with the terms [Cochlear Implants] limit to [human] limit to [English language] limit to [1995-2005] NOT [review articles] NOT [abstracts] AND [adult]. A total of 115 citations were retrieved for initial review. Many of these had been included with the requestor's submission. We excluded commentaries, letters, and case reports, as well as articles that did not address pre-implantation hearing tests in Medicare age patients. No additional articles were found.

Dalton et al, (2003) reported five-year follow-up data from the Epidemiology of Hearing Loss Study for 2688 patients with a mean age of 69 years. This provided an epidemiologic review of the extent of hearing problem in the Medicare population. By audiologic test data, 28% of the group had a mild hearing loss and 24% had a moderate to severe hearing loss. "Severity of hearing loss was significantly associated with having a hearing handicap and with self reported communication difficulties." Severity was associated with decreased SF-36 scores for mental and physical functioning.

Pasanisi et al, (2003) reviewed records of 105 patients implanted in a single institution over 11½ years. Nineteen of the patients were between 65 and 74 years old and data were reported for 16 members of the group who met criteria for duration of deafness, device used and who had at least one year follow-up. That group was compared to a reference group of 14 implanted subjects aged 41 to 59. "Before implantation the mean correct score for the two groups was close to 0% on both the closed-set and open-set tests." "...at 1 year after implantation...(a)ll of the subjects were full-time users of their implants." "The mean word recognition scores were 72.5% (range 46-100%; SD 16.76) for the elderly group and 82% (range 40-100%) for the reference group. No significant difference ($t = 1.444$, $P = 0.160$) was found for word recognition scores between the two groups. For everyday sentence recognition test, no significant difference between the elderly group scores and the reference group scores was detected ($t = 1.713$, $P = 0.098$, with means of 72.5% correct...and 85.7% correct...respectively."

Francis et al, (2002), using questionnaires and retrospective chart review, reported on 47 of 82 eligible patients aged 50 to 80 with a mean age of 63.4 (SD 8.6 years) who responded to a written request for information. Postoperative speech perception data were available from the charts of 29 patients and showed no significant difference between study participants and nonparticipants. Six months after surgery, postlingually deafened patients had a “significant increase in speech perception scores...(paired t test, $t = -6.9$ [$P < .0001$ for CID sentences; $t = -5.8$ [$P < .0001$] for monosyllabic words) with minimal additional gains noted between 6 months and 1 year.” Data on pre-implant scores were not given other than in bar graph format. Interpretation of that data indicate pre-implant percent correct scores for CID sentences of approximately 20% and scores of approximately 70% at 6 and 12 months. Also, a bar graph of monosyllabic word scores showed pre-implant scores of 8% and 35% and 38% at 6 months and 12 months respectively. Patients reported an improved quality of life and an increase in social activity. Forty-two patients reported they would again choose to undergo the procedure. The study included a cost-utility analysis which calculated \$9530 per quality-adjusted life-year.

Djalilian et al, (2002) compared results for 33 patients > 60 years of age to 61 patients 18 to 59 years old. “There were no statistically significant differences in improvements between the two patient populations on any audiological test.” With regard to responses to a quality of life questionnaire: “The only statistically significant differences between the elderly group and the younger adult population...were in recognizing a busy signal and recognizing a voice.” Ninety seven percent of younger patients as compared to 71% of older patients could recognize a busy signal and 91% of younger patients could recognize a voice compared to 56% of older patients. “There were no life-threatening complications in either patient population” with the older group experiencing two major complications (fistula and temporary facial paralysis) while the younger group had seven.

Labadie et al, (2000) reported on 36 patients, 16 of whom were 65 or older. Fifteen of the older patients had comorbid conditions. The elderly patients were found to “show significant benefit from cochlear implantation as assessed by audiologic testing” and had “no significant difference in operative time, length of stay in the hospital, hospital charges, and audiologic speech recognition scores” as compared to younger patients. CID sentence scores for both older and younger patients were again presented only in bar graph format with the following results: Older patients had pre-implant percent scores of approximately 23% and 3 and 6 month scores of 68% and 69%. Younger patients had pre-implant scores of 22% and 3 and 6 month scores of 77% and 75%.

Herzog et al. (2003) reported a three year study of 36 patients aged 65 – 85, comparing audiologic improvement after implantation to that of 101 patients aged 16 - 64. Although the older patients reached the same level of speech perception as the younger patients, the older ones needed a longer time after implantation to attain maximum perception. Perception of monosyllabic words plateaued at 55% at two years for both groups. At a signal to noise ratio of 15%, younger patients achieved the 55% perception at one year, while older patients required 4 years to do so.

Another important issue addressed in the material received was duration of deafness. The material included seven articles dealing with this topic including a 2003 study by Gomaa et al. that showed that for each year of deafness there was a 0.8% decline in post implant hearing scores and a 2002 study by Fetterman and Domico that showed negative correlation between length of deafness and performance.

With regard to the specific issue of the audiologic criteria that should be required for Medicare coverage of this procedure, the requestor provided a copy of the supporting evidence that was submitted to the FDA in connection with the November 1, 2000, approval of the Nucleus 24 Contour Cochlear Implant. Additionally, we reviewed the Summary of Safety and Effectiveness Data posted on the FDA website supporting the Nucleus 24 Cochlear Implant system, approved June 25, 1998.

The Summary of Safety and Effectiveness Data, which detailed the data that supported the change in audiologic criteria from $\leq 30\%$ to $\leq 40\%$ correct, states that data were collected on 67 of 135 implanted adults and that 98.5% (66/67) "...demonstrated significant improvement in the recognition of open-set sentences (CUNY) after 3 months of device use." Further, "(a)fter 3 months...recipients recognized an average of 78% of recorded sentences (CUNY) without lip reading. The median recorded sentence recognition score (CUNY) after 3 months of device use was 87%. Approximately one-half of the recipients (49.3%) recognized 90% or more of recorded word in sentences (CUNY) without lip-reading...Approximately two-thirds (62.7%) of recipients recognized 80% or more of recorded words in sentences (CUNY) without lip-reading after three months of device use." A number of other study results are included, all showing positive outcomes in these patients. Twelve month observational safety data on 133/135 implanted patients showed 20 minor complications, which resolved without surgery and two major complications, (hematoma and air leak) which required surgical correction. The Summary concludes that: "Significant improvements in scores for the majority of subjects demonstrated the effectiveness of the device in adult subjects."

The requestor also submitted the material that was used to support the supplemental request to change audiologic criteria from $\leq 40\%$ to $\leq 50\%$ correct scores. The data included 62 adult patients, 23 of whom were 65 years of age or older. There were several types of audiologic tests reported, but for most tests there were three month post-implantation data available on only seven of the ≥ 65 year old patients or 30% of recipients. One month data was available on 23 patients. Of the various tests given, 12 -16 of the 23 showed improvements of > 15 percentage points and 6 of the 7 showed a similar improvement at 3 months. However, only one of the patients had a pre-implant score of $> 40\%$.

Subsequently, the requestor submitted a list of forty patients with no information about age except that they were adults > 18 , with pre-operative open-set sentence discrimination scores between 30 and 50 percent. This information was obtained from ten implanting centers in response to CMS' request for more specific information than was previously provided. The requestor noted that a clinically significant change would be $\geq 15\%$ from pre-operative to most recent post-operative test results. The data showed that 25 patients had pre-implantation test scores between 30 and 40 percent and that all but one of these patients achieved at least a 15 % improvement on follow-up testing, occurring between 3 and 12 months after surgery.

The results for the fifteen patients with pre-implantation test scores between 41 and 50 percent showed improvements of > 15 percentage points in 11, worse post-implantation test scores for two patients, and two who failed to achieve the 15% improvement considered significant .

Table 1

Table 1

Table 1					
Source	Age	N	Pre scores	Post scores	Compared to younger group
Pasanisi	65-74	19	0%	72.50%	NS
Francis	50-80	47	20%	70%	NS
Djalilian	>60	33	-	-	NS QoL
Labadie	>65	16	23%	69%	NS
Herzog	65-85	36	?	55%	NS
FDA SS&E	Adults	66	30-40%*	78%	
FDA Supplemental	>65	1	40-50%	98%	
Requestor	Adults	25	30-40%	24 w/>15%	
		15	40-50%	11 w/>15%	

* Inferred, not specified in SS&E

4. Medicare Coverage Advisory Committee

The Medicare Coverage Advisory Committee was not convened to review this issue.

5. Evidence-based Guidelines

We could not, as of December 17, 2004, locate current evidence-based guidelines on cochlear implantation that include numeric audiologic criteria, nor were any suggested by the requestor.

6. Professional Society Position Statements

We reviewed the combined Veterans Administration (VA) and Department of Defense (DoD) Clinical Practice Recommendations for Prescription of Cochlear Implants. This document provided recommendations for their respective facilities but did not review evidence. It did include the following audiologic criteria: "Speech recognition ability for open-set speech meets FDA guidelines or IRB-approved protocol when veteran is fit with optimal amplification."

We reviewed the Aetna Clinical Policy Bulletin, #0013, 2/17/04, (Accessed December 17, 2004) which listed the following adult patient criteria:

"Member has limited benefit from appropriately fitted binaural hearing aids. Limited benefit from amplification is defined by test scores of 40 percent correct or less in best-aided listening condition on open-set sentence discrimination (e.g., CID sentences, Hearing in Noise Test sentences (HINT))."

The American Academy of Otolaryngology-Head and Neck Surgery stated that aligning CMS policy with the approved FDA criteria will provide Medicare beneficiaries with an opportunity to achieve better outcomes in a cost effective manner.

The American Speech-Language-Hearing Association (ASHA) referenced 1991 and 1995 studies that have shown that by adopting FDA's allowance of higher sentence recognition scores, more adults can benefit from cochlear implants.

The American Otological Society stated that coverage of cochlear implants for Medicare beneficiaries with words-in-sentences understanding scores of 30 to 50% correct with binaural hearing aids offers benefit that far outweighs associated risks and costs.

The American Academy of Audiology requested that CMS revise Medicare coverage language to reflect current FDA standards.

7. Expert Opinion

Except from communications with the NCD requestor, CMS received no formal expert opinion statements from the medical or scientific community regarding this issue.

8. Public Comments

During the public comment period on cochlear implants, July 8-Aug 9, 2004, CMS received 67 comments supporting the request to revise the national coverage determination on Cochlear Implants in accordance with the FDA guidelines. Over thirty of these comments represent universities and other institutional cochlear implant centers.

IX. CMS Analysis

National coverage determinations (NCDs) are determinations by the Secretary with respect to whether or not a particular item or service is covered nationally under title XVIII of the Social Security Act § 1869(f)(1)(B). In order to be covered by Medicare, an item or service must fall within one or more benefit categories contained within Part A or Part B, and must not be otherwise excluded from coverage. Moreover, with limited exceptions, the expenses incurred for items or services must be "reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member." § 1862(a)(1)(A). This section represents the agency's evaluation of the evidence considered and tentative conclusions reached for the assessment question.

Section 1862(a)(7) of the Social Security Act specifically precludes payment for expenses incurred for hearing aids. See also 42 C.F.R. § 411.15(d). However, cochlear implants are not hearing aids. Cochlear implants are prosthetic devices.

There are few published scientific articles addressing the audiologic criteria for cochlear implantation in Medicare-aged patients. We were unable to locate peer-reviewed published reports of randomized controlled trials containing pre-implantation and post-implantation audiologic data for patients 65 and older. Limited unpublished outcome data are available for analysis. In this instance, the pathophysiology of nerve deafness and the mechanism of action of the proposed treatment permit cautious inference of benefit based on published data from younger populations.

The majority of data are from the general adult population with pre-implant scores varying widely. The published data on the elderly population includes the entire range of pre-implant scores and demonstrate that the elderly have similar outcomes to younger age groups. The data submitted to the FDA for the approval of the 40% word recognition label included 66 adults without identifying their ages. In this group with an initial hearing score of $> 30\%$ to $\leq 40\%$, there was a significant improvement with a mean post-implantation score of 78%. None of the literature we reviewed indicated that surgical hazards of implantation in an elderly patient out-weighed the potential benefits of improved hearing and quality of life. Therefore, we believe that the evidence of similar outcomes seen in the elderly compared to younger populations in studies that include wide ranges of hearing loss, combined with the evidence on the group of patients (66) with specific pre-implants scores of $> 30\%$ to $\leq 40\%$ correct that demonstrated significant improvement with cochlear implant, though not robust, is compelling and sufficient to expand our coverage to this population.

There is insufficient data specifically dealing with older patients who had pretest scores of $> 40\%$ to $\leq 50\%$. We were only able to identify one adult age 65 or older in published data with these scores. And, in the unpublished data supplied by the requestor, the ages of the adult population were not specified and only 15 had a pre-implant score of $> 40\%$ to $\leq 50\%$. However, of that group, 11/15 showed improvement. We are not confident that this is a now a sufficient evidence base to warrant unmonitored expansion of coverage beyond the 40% level. We are concerned that the available evidence does not allow providers to target these devices to patients who will clearly derive benefit. In order to provide maximum protection to our beneficiaries, CMS will require that reimbursement for cochlear implants for beneficiaries with a pre-implant score of $> 40\%$ to $\leq 60\%$ be made only for patients enrolled in either a FDA approved category B IDE clinical trial, a trial under the CMS Clinical Trial Policy, or a prospective, controlled comparative trial approved by CMS as consistent with the evidentiary requirements for National Coverage Analyses (See Appendix B) and meeting specific quality standards (See Appendix C). We believe that expanding the pre-implant score to 60% will also assist in determining if there is a broader population of Medicare beneficiaries for whom the cochlear implant is reasonable and necessary while at the same time providing the protection of a clinical trial.

The submission of data on patients receiving a cochlear implant to a data collection process is reasonable and necessary to assure patient safety and protection. Data will help identify the appropriate patient populations and provide patient protections and safeguards that would only be available to the extent that data can be made available in some form to providers and practitioners to inform their decisions, monitor performance quality, benchmark and identify best practices.

IX. Proposed Conclusions

The Centers for Medicare and Medicaid Services (CMS) is seeking public comment on the following proposed conclusions:

The evidence is adequate to conclude that cochlear implantation is reasonable and necessary for treatment of bilateral pre-or-postlinguistic, sensorineural moderate-to-profound hearing loss in individuals who demonstrate limited benefit from amplification. Limited benefit from amplification is defined by test scores of $\leq 40\%$ correct in the best-aided listening condition on tape recorded tests of open-set sentence cognition.

The evidence is sufficient to conclude that a cochlear implant is reasonable and necessary for individuals with hearing test scores of $> 40\%$ and $\leq 60\%$ only when the provider is participating in and patients are enrolled in either an FDA-approved category B IDE clinical trial, a trial under the CMS Clinical Trial Policy, or a prospective, controlled comparative trial approved by CMS as consistent with the evidentiary requirements for National Coverage Analyses (See Appendix B) and meeting specific quality standards (See Appendix C).

CMS is requesting public comments on this proposed decision memorandum pursuant to Section 731 of the Medicare Modernization Act. After considering the public comments and any additional evidence, we will make a final determination and issue a final coverage decision memorandum

APPENDIX A

References

APPENDIX B

General Methodological Principles of Study Design (Section VI of the Decision Memorandum)

When making national coverage determinations, CMS evaluates relevant clinical evidence to determine whether or not the evidence is of sufficient quality to support a finding that an item or service is reasonable and necessary. The overall objective for the critical appraisal of the evidence is to determine to what degree we are confident that: 1) the specific assessment questions can be answered conclusively; and 2) the intervention will improve net health outcomes for patients.

We divide the assessment of clinical evidence into three stages: 1) the quality of the individual studies; 2) the generalizability of findings from individual studies to the Medicare population; and 3) overarching conclusions that can be drawn from the body of the evidence on the direction and magnitude of the intervention's potential risks and benefits.

The methodological principles described below represent a broad discussion of the issues we consider when reviewing clinical evidence. However, it should be noted that each coverage determination has its unique methodological aspects.

Assessing Individual Studies

Methodologists have developed criteria to determine weaknesses and strengths of clinical research. Strength of evidence generally refers to: 1) the scientific validity underlying study findings regarding causal relationships between health care interventions and health outcomes; and 2) the reduction of bias. In general, some of the methodological attributes associated with stronger evidence include those listed below:

- Use of randomization (allocation of patients to either intervention or control group) in order to minimize bias.
- Use of contemporaneous control groups (rather than historical controls) in order to ensure comparability between the intervention and control groups.
- Prospective (rather than retrospective) studies to ensure a more thorough and systematic assessment of factors related to outcomes.
- Larger sample sizes in studies to demonstrate both statistically significant as well as clinically significant outcomes that can be extrapolated to the Medicare population. Sample size should be large enough to make chance an unlikely explanation for what was found.
- Masking (blinding) to ensure patients and investigators do not know to which group patients were assigned (intervention or control). This is important especially in subjective outcomes, such as pain or quality of life, where enthusiasm and psychological factors may lead to an improved perceived outcome by either the patient or assessor.

Regardless of whether the design of a study is a randomized controlled trial, a non-randomized controlled trial, a cohort study or a case-control study, the primary criterion for methodological strength or quality is the extent to which differences between intervention and control groups can be attributed to the intervention studied. This is known as internal validity. Various types of bias can undermine internal validity. These include:

- Different characteristics between patients participating and those theoretically eligible for study but not participating (selection bias).
- Co-interventions or provision of care apart from the intervention under evaluation (performance bias).
- Differential assessment of outcome (detection bias).
- Occurrence and reporting of patients who do not complete the study (attrition bias).

In principle, rankings of research design have been based on the ability of each study design category to minimize these biases. A randomized controlled trial minimizes systematic bias (in theory) by selecting a sample of participants from a particular population and allocating them randomly to the intervention and control groups. Thus, in general, randomized controlled studies have been typically assigned the greatest strength, followed by non-randomized clinical trials and controlled observational studies. The design, conduct and analysis of trials are important factors as well. For example, a well designed and conducted observational study with a large sample size may provide stronger evidence than a poorly designed and conducted randomized controlled trial with a small sample size. The following is a representative list of study designs (some of which have alternative names) ranked from most to least methodologically rigorous in their potential ability to minimize systematic bias:

- Randomized controlled trials
- Non-randomized controlled trials
- Prospective cohort studies
- Retrospective case control studies
- Cross-sectional studies
- Surveillance studies (e.g., using registries or surveys)
- Consecutive case series
- Single case reports

When there are merely associations but not causal relationships between a study's variables and outcomes, it is important not to draw causal inferences. Confounding refers to independent variables that systematically vary with the causal variable. This distorts measurement of the outcome of interest because its effect size is mixed with the effects of other extraneous factors. For observational, and in some cases randomized controlled trials, the method in which confounding factors are handled (either through stratification or appropriate statistical modeling) are of particular concern. For example, in order to interpret and generalize conclusions to our population of Medicare patients, it may be necessary for studies to match or stratify their intervention and control groups by patient age or co-morbidities.

Methodological strength is, therefore, a multidimensional concept that relates to the design, implementation and analysis of a clinical study. In addition, thorough documentation of the conduct of the research, particularly study selection criteria, rate of attrition and process for data collection, is essential for CMS to adequately assess and consider the evidence.

Generalizability of Clinical Evidence to the Medicare Population

The applicability of the results of a study to other populations, settings, treatment regimens and outcomes assessed is known as external validity. Even well designed and well-conducted trials may not supply the evidence needed if the results of a study are not applicable to the Medicare population. Evidence that provides accurate information about a population or setting not well represented in the Medicare program would be considered but would suffer from limited generalizability.

The extent to which the results of a trial are applicable to other circumstances is often a matter of judgment that depends on specific study characteristics, primarily the patient population studied (age, sex, severity of disease and presence of co-morbidities) and the care setting (primary to tertiary level of care, as well as the experience and specialization of the care provider). Additional relevant variables are treatment regimens (dosage, timing and route of administration), co-interventions or concomitant therapies, and type of outcome and length of follow-up.

The level of care and the experience of the providers in the study are other crucial elements in assessing a study's external validity. Trial participants in an academic medical center may receive more or different attention than is typically available in non-tertiary settings. For example, an investigator's lengthy and detailed explanations of the potential benefits of the intervention and/or the use of new equipment provided to the academic center by the study sponsor may raise doubts about the applicability of study findings to community practice.

Given the evidence available in the research literature, some degree of generalization about an intervention's potential benefits and harms is invariably required in making coverage determinations for the Medicare population. Conditions that assist us in making reasonable generalizations are biologic plausibility, similarities between the populations studied and Medicare patients (age, sex, ethnicity and clinical presentation) and similarities of the intervention studied to those that would be routinely available in community practice.

A study's selected outcomes are an important consideration in generalizing available clinical evidence to Medicare coverage determinations. One of the goals of our determination process is to assess net health outcomes. These outcomes include resultant risks and benefits such as increased or decreased morbidity and mortality. In order to make this determination, it is often necessary to evaluate whether the strength of the evidence is adequate to draw conclusions about the direction and magnitude of each individual outcome relevant to the intervention under study. In addition, it is important that an intervention's benefits are clinically significant and durable, rather than marginal or short-lived.

If key health outcomes have not been studied or the direction of clinical effect is inconclusive, we may also evaluate the strength and adequacy of indirect evidence linking intermediate or surrogate outcomes to our outcomes of interest.

Assessing the Relative Magnitude of Risks and Benefits

In general, an intervention is not reasonable and necessary if its risks outweigh its benefits. Among other things, CMS considers whether reported benefits translate into improved net health outcomes. CMS places greater emphasis on health outcomes actually experienced by patients, such as quality of life, functional status, duration of disability, morbidity and mortality, and less emphasis on outcomes that patients do not directly experience, such as intermediate outcomes, surrogate outcomes, and laboratory or radiographic responses. The direction, magnitude, and consistency of the risks and benefits across studies are also important considerations. Based on the analysis of the strength of the evidence, CMS assesses the relative magnitude of an intervention or technology's benefits and risk of harm to Medicare beneficiaries.

Appendix C

Standards of Qualifying Clinical Trials

During implementation of its current NCD on clinical trials⁴, CMS asked the Agency for Healthcare Research and Quality (AHRQ) to consult with a multi-agency panel in order to develop a set of criteria CMS could use to identify clinical trials that should receive Medicare coverage. AHRQ convened a panel composed of representatives from the FDA, National Institutes of Health, Centers for Disease Control and Prevention, Department of Defense, Veteran's Administration, and the Department of Health and Human Services (DHHS) Office for Human Research and Protection. This panel held several meetings, including two public meetings in which interested parties were given the opportunity to provide comments.⁵ Based on the above activities, CMS considers the following to be the specific quality standards of a clinical trial:

A. Required Elements of the Written Protocol ⁶

1.

The principal investigator must certify that he/she or the fiscal officer of his/her institution will keep a copy of the final written protocol on file and, upon request, make it available to CMS.

2.

An abstract of the written protocol will be submitted as part of the registration process.

3. The written protocol must include the following information:

- a. Identifying information
- b. Scientific background
- c. Objectives and hypothesis
- d. Design
- e. Criteria for selection, exclusion, and withdrawal of subjects
- f. Interventions (where applicable) and other treatments for subjects under each arm of the study
- g. Outcome measures
- h. Statistical analysis plan
- i. Discussion of quality control, data management, and record keeping procedures, including plans to ensure compliance with prevailing privacy regulations
- j. Conflict of interest policies
 - i. If the research is being conducted at an institution with a conflict of interest policy, this should be noted, with a statement that the policies are being followed;
 - ii. If there are no institutional conflict of interest policies, then the protocol should identify a set of policies that are being used; options include: - U.S Public Health Service regulations: 42 CFR Part 50 Sec. 50.604; Institutional responsibility regarding conflicting interests of investigators:

(http://www.access.gpo.gov/nara/cfr/waisidx_00/42cfr50_00.html).

- Association of American Medical Colleges Guidelines for Dealing with Faculty Conflicts of Commitment and Conflicts of Interest in Research:

(<http://www.aamc.org/research/dbr/coi.htm>).

- American Medical Association Guidelines for Conflicts of Interest in Biomedical Research and Health Facility Ownership by a Physician:

(<http://www.ama-assn.org/ethic/ceja/report95.pdf>) and

(<http://www.amaassn.org/ethic/ceja/06b.pdf>), respectively.

- k. Other ethical issues, where applicable
- l. Publication policy:
 - i. Protocol should describe the specific publication policies that are being followed.
 - ii. Principal investigator (P1) must certify that: -Investigators have the right to publish findings from this trial without receiving approval from the trial's financial sponsors.⁶⁵
-Investigators agree to notify ClinicalTrials.Gov of initial publications based on data from this trial.

B. Institutional Review Board (IRB) review and approval

1. The principal investigator must certify that an IRB has reviewed and approved the trial. Evidence of this must be kept on file, and be made available to the Secretary for review on request.

2. Although the term IRB has been used to describe a range of committees, the use of the term here refers to a committee that is constituted and operates in a manner consistent with the definition and procedures specified in Department of Health and Human Services (DHHS) Regulations for the Protection of Human Subjects in the Code of Federal Regulations (45CFR Part 46).⁷

3. The Office for Human Research Protection (OHRP) is taking several steps that are designed to enhance the functioning of IRBs. These steps include developing a system of IRB registration and implementing a streamlined assurance program. In addition, IRB accreditation programs are being explored (and in the case of the VA, implemented). All of these steps are important to enhance the functioning of IRBs, and the panel believes that they should be required as part of the Medicare qualifying criteria as soon as appropriate systems are in place.

C. Scientific Review and Approval ⁸

1.

Review of a trial protocol by two or more qualified individuals who are not part of the research team is important to ensure that the trial has scientific merit.

2. Critical elements of scientific review include the following:

- a. Importance and relevance of the research question(s)
- b. Soundness of the study's scientific rationale
- c. Previous research to support proceeding to clinical trials in human beings (if appropriate)
- d. Adequacy of the study design and procedures to evaluate the specific research question(s)
- e. Appropriateness of the study population (e.g., age, gender, health status)
- f. Appropriateness of statistical plan
- g. Feasibility of carrying out the study
- h. Qualifications of the investigators
- i. Evidence and assurance that risks to human subjects are minimized

3. Two or more individuals who have the appropriate range of expertise must conduct the scientific review (including clinical trial methodology and content area of the trial). The individuals who conduct the review should not have direct involvement with the research team, and should not have direct financial ties to or interests in the research. The review may be conducted by a standing scientific review committee or by two or more individuals identified by the principal investigator. The principal investigator must specify the names and contact information of the reviewers (or the standing committee and its chair) and the date of approval.

D. Certification that investigators have not been disqualified

The principal investigator must certify that none of the trial investigators have been barred from participating in human subjects research by the FDA, Office of Research Integrity (ORI), Office for Human Research Protections (OHRP), or any other Federal agency. The principal investigator must inform CMS if any investigator becomes disqualified over the course of the trial.

¹ US Food and Drug Administration. Straight Talk from FDA About Hearing Loss and Hearing Aids. March 2002

² Cochlear Implants Fact Sheet. American Speech-Language-Hearing Association website

³ National Institutes of Health. NIH Consensus Statement. Vol 13, No.2, May 15-17 1995

⁴ <http://www.cms.hhs.gov/coverage/8d3.asp>

⁵ FR Notice for October 20, 2000 Meeting. Federal Register. October 11, 2000 (Volume 65. Number 197)

⁶ 64 Adapted from the following: 1) International Conference on the Harmonization of Technical Requirements for Registration of Pharmaceutical for Human Use: Guideline for GoodClinicalPractice.May1996.

(http://www.ich.org/MediaServer.jserv?@_ID=482&@_MODE=GLB); 2)NIH scientific review group evaluations of clinical protocols:

(<http://grants.nih.gov/grants/guide/notice-files/not97-010.html>); and 3)Elements of an NCI request for a proposal

(<http://rcb.nci.nih.gov/appl/rfp/85080/SOWMain.htm>).

⁷ Code of Federal Regulations: Title 45 Public Welfare Department of Health and Human Services, Part 46:

⁸ Adapted from the following: 1) Hellen Gelband. A Report on the Sponsors of Cancer Treatment Clinical Trials and their Approval and Monitoring Mechanisms; prepared for the National Cancer Policy Board. February, 1999; 2) NIH scientific review group evaluations of clinical protocols:

(<http://grants.nih.gov/grants/guide/notice-files/not97-010.html>); and 3) NHLBI guidelines for submission of investigator initiated clinical protocols:

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